

1060 1070 1080 1090 1100 1110 1120
 * * * * * * *
 ATACCAAGGAC ATTGTATGAC GTCCTCTGCT CACATGCCCTG CTTTCTTCCT ATAATACAGA TGCTCAACTA
 * * * * * * *
 1130 1140 1150 1160 1170 1180 1190
 * * * * * * *
 ACTGCTCATG TCCTTATATC ACAGAGGGAA ATTGGAGCTA TCTGAGGAAC TGCCCAGAAC GGAAGGGCAG
 * * * * * * *
 1200 1210 1220 1230 1240 1250 1260
 * * * * * * *
 AGGGGTCTTG CTCTCCTTGT CTGAGCCATA ACTCTTCTTT CTACCTTCCA GTGAACACCT TCCCACCCCCA
 * * * * * * *
 1270 1280 1290 1300 1310 1320 1330
 * * * * * * *
 GGTCCACCTG CTACCGCCGC CGTCGGAGGA GCTGGCCCTG AATGAGCTCT TGTCCCTGAC ATGCCTGGTG
 * * * * * * *
 1340 1350 1360 1370 1380 1390 1400
 * * * * * * *
 CGAGCTTTCA ACCCTAAAGA AGTGCTGGTG CGATGGCTGC ATGGAAATGA GGAGCTGTCC CCAGAAAGCT
 * * * * * * *
 1410 1420 1430 1440 1450 1460 1470
 * * * * * * *
 ACCTAGTGTGTT TGAGCCCCCTA AAGGAGCCAG GCGAGGGAGC CACCACCTAC CTGGTGACAA GGTGTTGCG
 * * * * * * *
 1480 1490 1500 1510 1520 1530 1540
 * * * * * * *
 TGTATCAGCT GAAAGCTTGA TATCGAATTG CCGAGGGCGGA ACCGGCAGTG CAGCCCGAAG CCCCGCAGTC
 * * * * * * *
 1550 1560 1570 1580 1590 1600 1610
 * * * * * * *
 CCCGAGCACG CGTGGCC ATG CGT CCC CTG CGC CCC CGC GCC GCG CTG CTG GCG CTC CTG
 Met Arg Pro Leu Arg Pro Arg Ala Ala Leu Leu Ala Leu Leu Leu
 _____a____a____a____a____a____a____ORF RF[1] _____a____a____a____a____a____a____>
 1600 1610 1620 1630 1640 1650
 * * * * * *
 GCC TCG CTC CTG GCC GCG CCC CCG GTG GCC CCG GCC GAG GCC CCG CAC CTG GTG CAT
 Ala Ser Leu Leu Ala Ala Pro Pro Val Ala Pro Ala Glu Ala Pro His Leu Val His
 _____a____a____a____a____a____a____a____ORF RF[1] _____a____a____a____a____a____a____>
 1660 1670 1680 1690 1700 1710
 * * * * * *
 GTG GAC GCG GCC CGC GCG CTG TGG CCC CTG CGG CGC TTC TGG AGG AGC ACA GGC TTC
 Val Asp Ala Ala Arg Ala Leu Trp Pro Leu Arg Arg Phe Trp Arg Ser Thr Gly Phe
 _____a____a____a____a____a____a____a____ORF RF[1] _____a____a____a____a____a____a____>
 1720 1730 1740 1750 1760 1770
 * * * * * *
 TGC CCC CCG CTG CCA CAC AGC CAG GCT GAC CAG TAC GTC CTC AGC TGG GAC CAG CAG
 Cys Pro Pro Leu Pro His Ser Gln Ala Asp Gln Tyr Val Leu Ser Trp Asp Gln Gln
 _____a____a____a____a____a____a____a____ORF RF[1] _____a____a____a____a____a____a____>
 1780 1790 1800 1810 1820
 * * * * * *
 CTC AAC CTC GCC TAT GTG GGC GCC GTC CCT CAC CGC GGC ATC AAG CAG GTC CGG ACC
 Leu Asn Leu Ala Tyr Val Gly Ala Val Pro His Arg Gly Ile Lys Gln Val Arg Thr
 _____a____a____a____a____a____a____a____ORF RF[1] _____a____a____a____a____a____a____>
 1830 1840 1850 1860 1870 1880
 * * * * * *
 CAC TGG CTG CTG GAG CTT GTC ACC ACC AGG GGG TCC ACT GGA CGG GGC CTG AGC TAC
 His Trp Leu Leu Glu Leu Val Thr Thr Arg Gly Ser Thr Gly Arg Gly Leu Ser Tyr
 >

FIGURE 1A

FIGURE 1B.

FIGURE 1C

FIGURE 1D

AAAAAAAA AAAAAAAAG AATTCTGCA GCCCGGGGA TCCACTAGTT CTAGAGGGCC CGTTTAAACC
 3760 3770 3780 3790 3800 3810 3820
 * * * * * * * *
 CGCTGATCAG CCTCGACTGT GCCTTCTAGT TGCCAGCCAT CTGTTGTTG CCCCTCCCCC GTGCCTTCCT
 3830 3840 3850 3860 3870 3880 3890
 * * * * * * * *
 TGACCCCTGGA AGGTGCCACT CCCACTGTCC TTTCTAATA AAATGAGGAA ATTGCATCGC ATTGTCTGAG
 3900 3910 3920 3930 3940 3950 3960
 * * * * * * * *
 TAGGTGTCAT TCTATTCTGG GGGGTGGGGT GGGGCAGGAC AGCAAGGGG AGGATTGGGA AGACAATAGC
 3970 3980 3990 4000 4010 4020 4030
 * * * * * * * *
 AGGCATGCTG GGGATGCGGT GGGCTCTATG GCTTCTGAGG CGAAAGAAC CAGCTGGGC TCGAGAGCTT
 4040 4050 4060 4070 4080 4090 4100
 * * * * * * * *
 GGCGTAATCA TGGTCATAGC TGTTTCTGT GTGAAATTGT TATCCGCTCA CAATTCCACA CAACATACGA
 4110 4120 4130 4140 4150 4160 4170
 * * * * * * * *
 GCCCGAAGCA TAAAGTGTAA AGCCTGGGT GCCTAATGAG TGAGCTAACT CACATTAATT GCGTTGCGCT
 4180 4190 4200 4210 4220 4230 4240
 * * * * * * * *
 CACTGCCCGC TTTCCAGTCG GGAAACCTGT CGTGCCAGCT GCATTAATGA ATCGGCCAAC GCGCGGGAG
 4250 4260 4270 4280 4290 4300 4310
 * * * * * * * *
 AGGCCTGGTTG CGTATTGGGC GCTCTTCCGC TTCCCTGCTC ACTGACTCGC TGCGCTCGGT CGTCGGCTG
 4320 4330 4340 4350 4360 4370 4380
 * * * * * * * *
 CGGCCAGCGG TATCAGCTCA CTCAAAGGCC GTAATACGGT TATCCACAGA ATCAGGGGAT AACGCAGGAA
 4390 4400 4410 4420 4430 4440 4450
 * * * * * * * *
 AGAACATGTG AGCAAAAGGC CAGCAAAAGG CCAGGAACCG TAAAAAGGCC GCGTTGCTGG CGTTTTCCA
 4460 4470 4480 4490 4500 4510 4520
 * * * * * * * *
 TAGGCTCCGC CCCCTGACG AGCATCACAA AAATGACGC TCAAGTCAGA GGTGGCAGAA CCCGACAGGA
 4530 4540 4550 4560 4570 4580 4590
 * * * * * * * *
 CTATAAGAT ACCAGGCAGTT TCCCCCTGGA AGCTCCCTCG TGCGCTCTCC TGTTCCGACC CTGCCGCTTA
 4600 4610 4620 4630 4640 4650 4660
 * * * * * * * *
 CCGGATACCT GTCCGCCTTT CTCCCTTCGG GAAGCGTGGC GCTTTCTCAA TGCTCACGCT GTAGGTATCT
 4670 4680 4690 4700 4710 4720 4730
 * * * * * * * *
 CAGTTCGGTG TAGGTCGTTG GCTCCAAGCT GGGCTGTGTG CACGAACCCC CCGTTCAGCC CGACCGCTGC
 4740 4750 4760 4770 4780 4790 4800
 * * * * * * * *
 GCCTTATCCG GTAACTATCG TCTTGAGTCG AACCCGGTAA GACACGACTT ATGCCACTG GCAGCAGCCA

FIGURE 1E

4810	4820	4830	4840	4850	4860	4870
*	*	*	*	*	*	*
CTGGTAACAG GATTAGCAGA GCGAGGTATG TAGGCAGTGC TACAGAGTTC TTGAAGTGGT GGCTAACTA						
4880	4890	4900	4910	4920	4930	4940
*	*	*	*	*	*	*
CGGCTACACT AGAAGGACAG TATTTGGTAT CTGCAGCTTG CTGAAGCCAG TTACCTTCGG AAAAAGAGTT						
4950	4960	4970	4980	4990	5000	5010
*	*	*	*	*	*	*
GGTAGCTCTT GATCCGGCAA ACACACCACC GCTGGTAGCG GTGGTTTTTG TGTTTGCAAG CAGCAGATTA						
5020	5030	5040	5050	5060	5070	5080
*	*	*	*	*	*	*
CGCGCAGAAA AAAAGGATCT CAAGAAGATC CTTTGATCTT TTCTACGGGG TCTGACGCTC AGTGGAACGA						
5090	5100	5110	5120	5130	5140	5150
*	*	*	*	*	*	*
AAACTCACGT TAAGGGATTG TGGTCATGAG ATTATCAGAAA AGGATCTTCA CCTAGATCCT TTTAAATTAA						
5160	5170	5180	5190	5200	5210	5220
*	*	*	*	*	*	*
AAATGAAGTT TTAAATCAAT CTAAAGTATA TATGAGTAAA CTTGGTCTGA CAGTTACCAA TGCTTAATCA						
5230	5240	5250	5260	5270	5280	5290
*	*	*	*	*	*	*
GTGAGGCACC TATCTCAGCG ATCTGTCTAT TTCGTTCATC CATAAGTGCCT TGACTCCCCG TCGTGTAGAT						
5300	5310	5320	5330	5340	5350	5360
*	*	*	*	*	*	*
AACTACGATA CGGGAGGGCT TACCATCTGG CCCCAGTGCT GCAATGATAC CGCGAGACCC ACGCTCACCG						
5370	5380	5390	5400	5410	5420	5430
*	*	*	*	*	*	*
GCTCCAGATT TATCAGCAAT AAACCAGCCA GCCGGAAGGG CCCAGCGCAG AAGTGGTCCT GCAACTTTAT						
5440	5450	5460	5470	5480	5490	5500
*	*	*	*	*	*	*
CCGCCTCCAT CCAGTCTATT AATTGTTGCC GGGAAAGCTAG AGTAAGTAGT TCGCCAGTTA ATAGTTTGC						
5510	5520	5530	5540	5550	5560	5570
*	*	*	*	*	*	*
CAACGTTGTT GCCATTGCTA CAGGCATCGT GGTGTCACGC TCGTCGTTTG GTATGGCTTC ATTCAAGCTCC						
5580	5590	5600	5610	5620	5630	5640
*	*	*	*	*	*	*
GGTCCCCAAC GATCAAGGCG AGTTACATGA TCCCCCATGT TGTGCAAAAA AGCGGTTAGC TCCTTCGGTC						
5650	5660	5670	5680	5690	5700	5710
*	*	*	*	*	*	*
CTCCGATCGT TGTCAGAAAGT AAGTTGGCCG CAGTGTATC ACTCATGGTT ATGGCAGCAC TGCATAATTC						
5720	5730	5740	5750	5760	5770	5780
*	*	*	*	*	*	*
TCTTACTGTC ATGCCATCCG TAAGATGCTT TTCTGTGACT GGTGAGTACT CAACCAAGTC ATTCTGAGAA						
5790	5800	5810	5820	5830	5840	5850
*	*	*	*	*	*	*
TAGTGTATGC GGCGACCGAG TTGCTCTTGC CCGCGTCAA TACGGGATAA TACCGCGCCA CATAGCAGAA						
5860	5870	5880	5890	5900	5910	5920
*	*	*	*	*	*	*

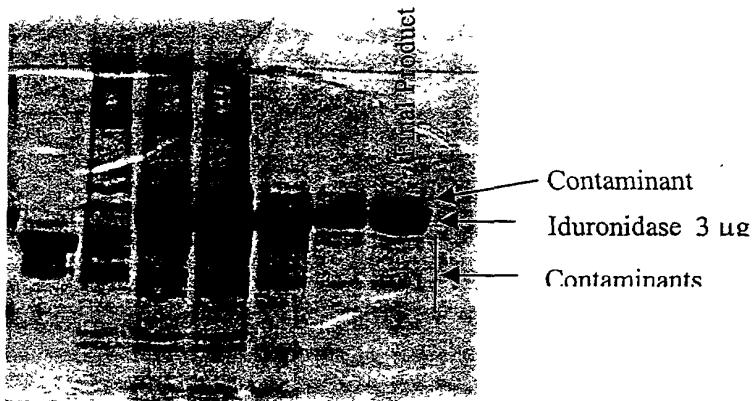
FIGURE 1F

CTTTAAAAGT GCTCATCATT GGAAAACGTT CTTCGGGGCG AAAACTCTCA AGGATCTTAC CGCTGTTGAG
5930 5940 5950 5960 5970 5980 5990
* * * * * * * * * * * *
ATCCAGTTCG ATGTAACCCA CTCGTGCACC CAACTGATCT TCAGCATCTT TTACTTTCAC CAGCGTTCT
6000 6010 6020 6030 6040 6050 6060
* * * * * * * * * * * *
GGGTGAGCAA AACAGGAAG GCAAAATGCC GCAAAAAAGG GAATAAGGGC GACACGGAAA TGTTGAATAC
6070 6080 6090 6100 6110 6120 6130
* * * * * * * * * * * *
TCATACTCTT CCTTTTCAA TATTATTGAA GCATTTATCA GGGTTATTGT CTCATGAGCG GATACATATT
6140 6150 6160 6170 6180 6190 6200
* * * * * * * * * * * *

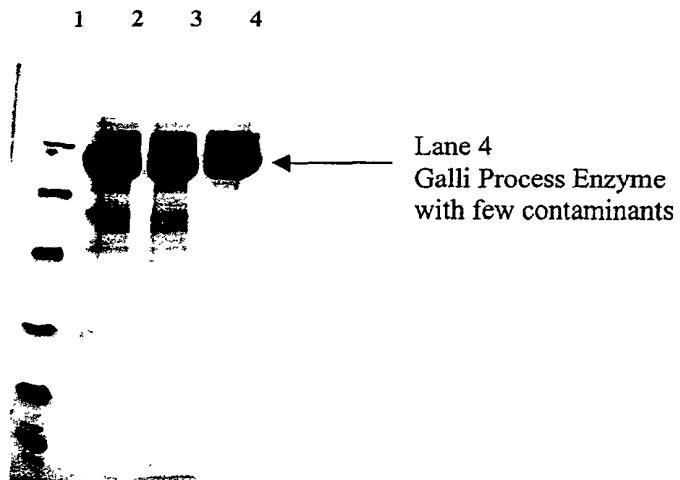
FIGURE 1G

FIGURE 2. SDS-POLYACRYLAMIDE GELS DEMONSTRATING IMPROVEMENTS IN PURITY

Gel using the Kakkis et al 1994, published procedure for purification



Gel using the new Galli Process contained in this application



1. Molecular Weight Marker
2. Prior Process Carson (nonpublished) Batch 2000C9001 Reference Reduced (7.5 µg)
3. Same Batch 2000C9001 Reference Reduced (5.0 µg)
4. Galli Process Enzyme Batch P10006 (5.0 µg)

FIGURE 2

FIGURE 3A IDURONIDASE PRODUCTION USING THE GALLI PROCESS

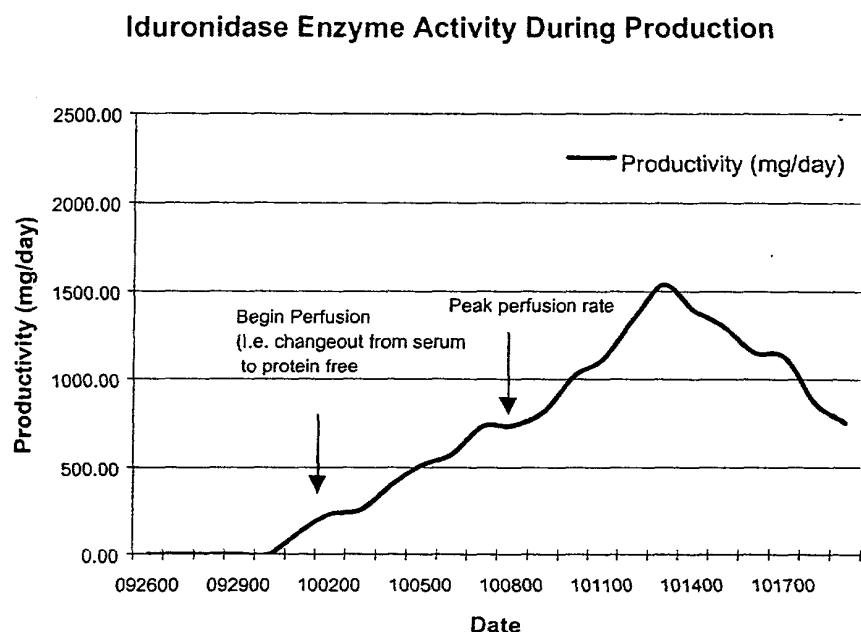
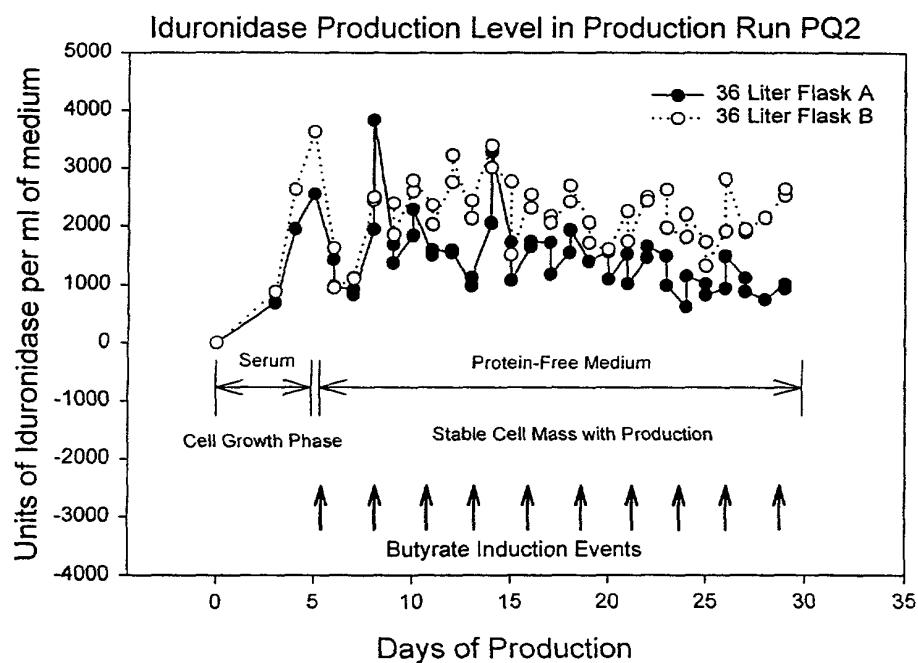


FIGURE 3B. IDURONIDASE PRODUCTION USING BUTYRATE INDUCTION



Reduction in Liver Volume During Enzyme Therapy

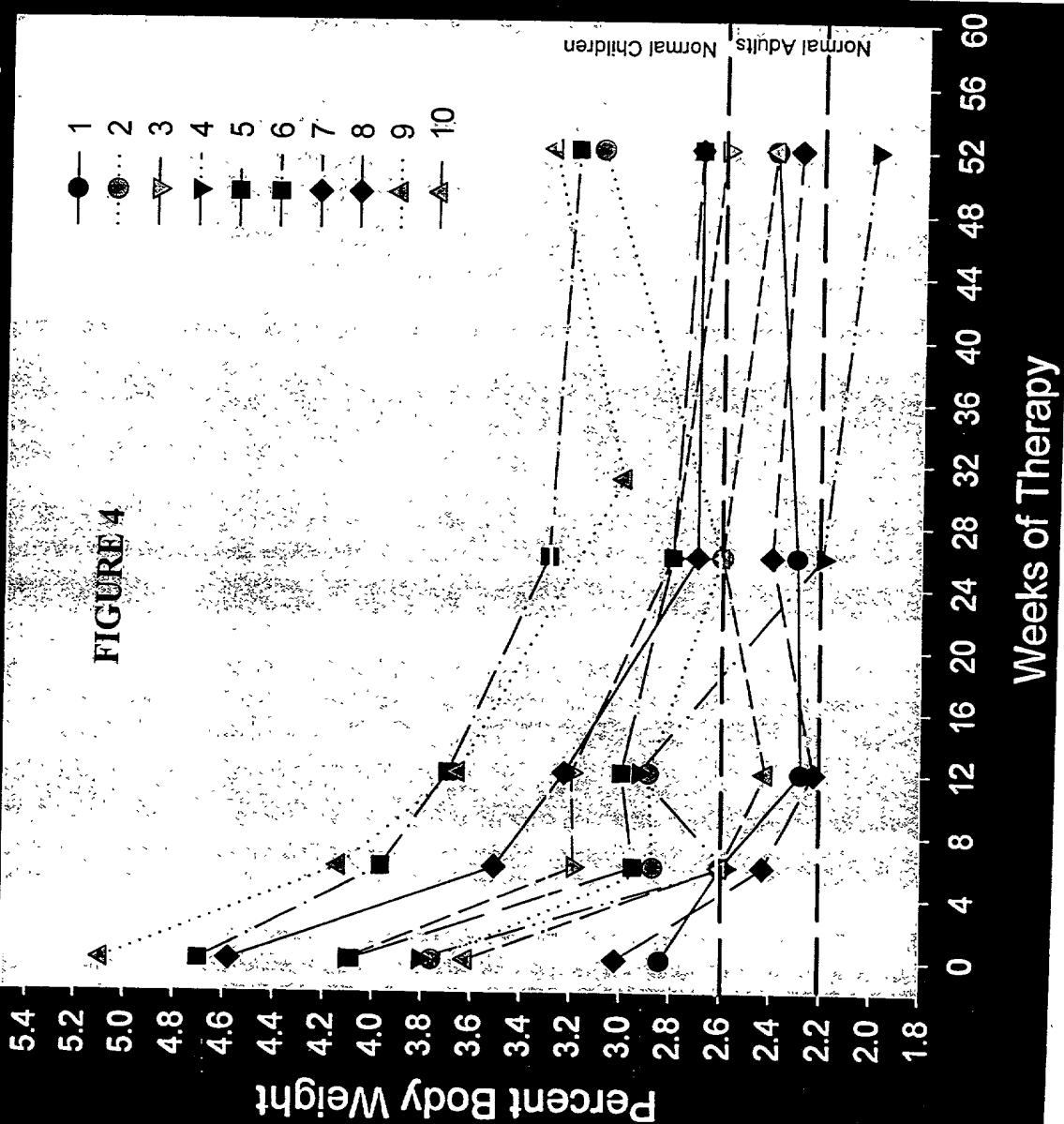


FIGURE 4

Urinary GAG Excretion During Enzyme Therapy

FIGURE 5

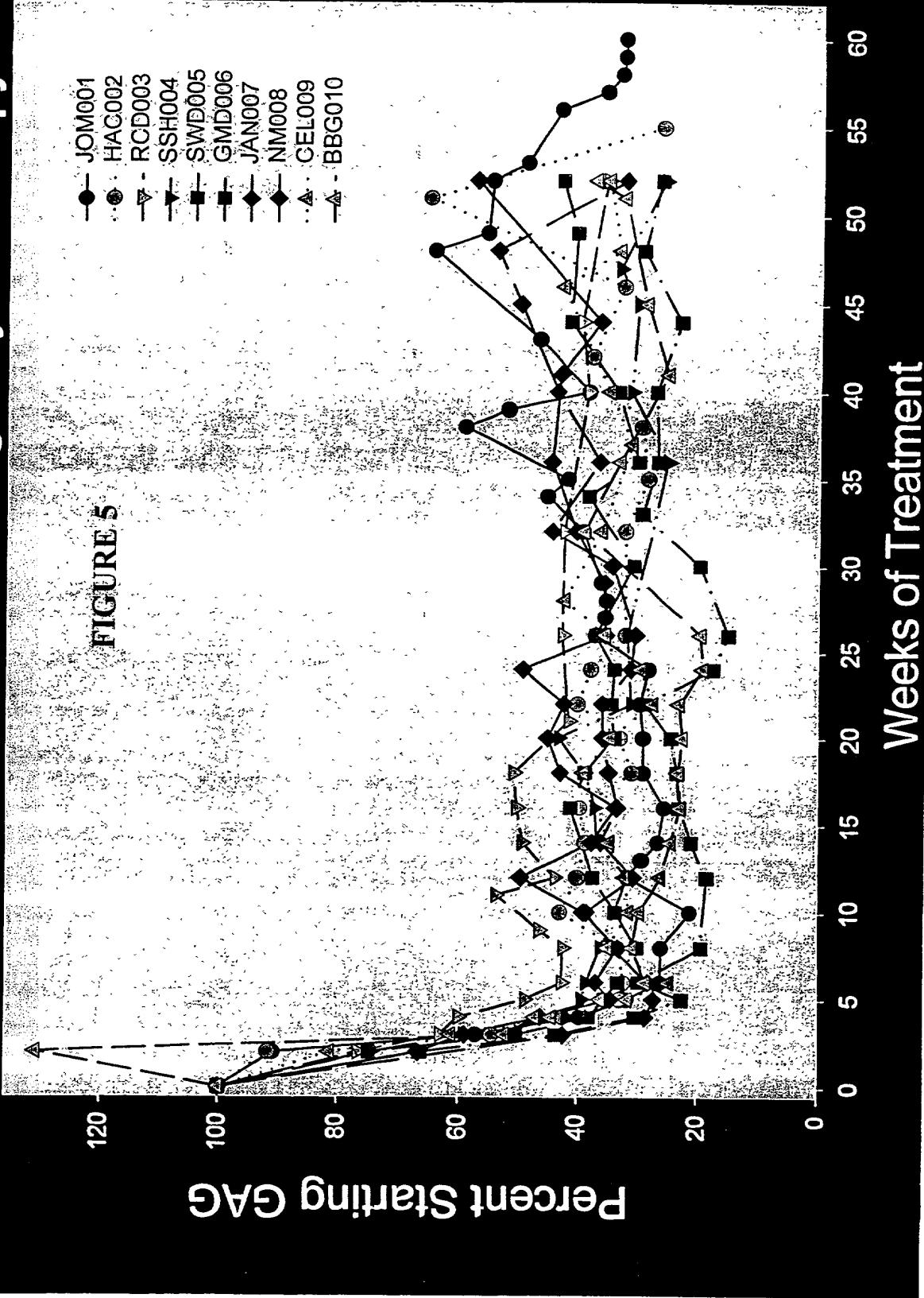


FIGURE 5

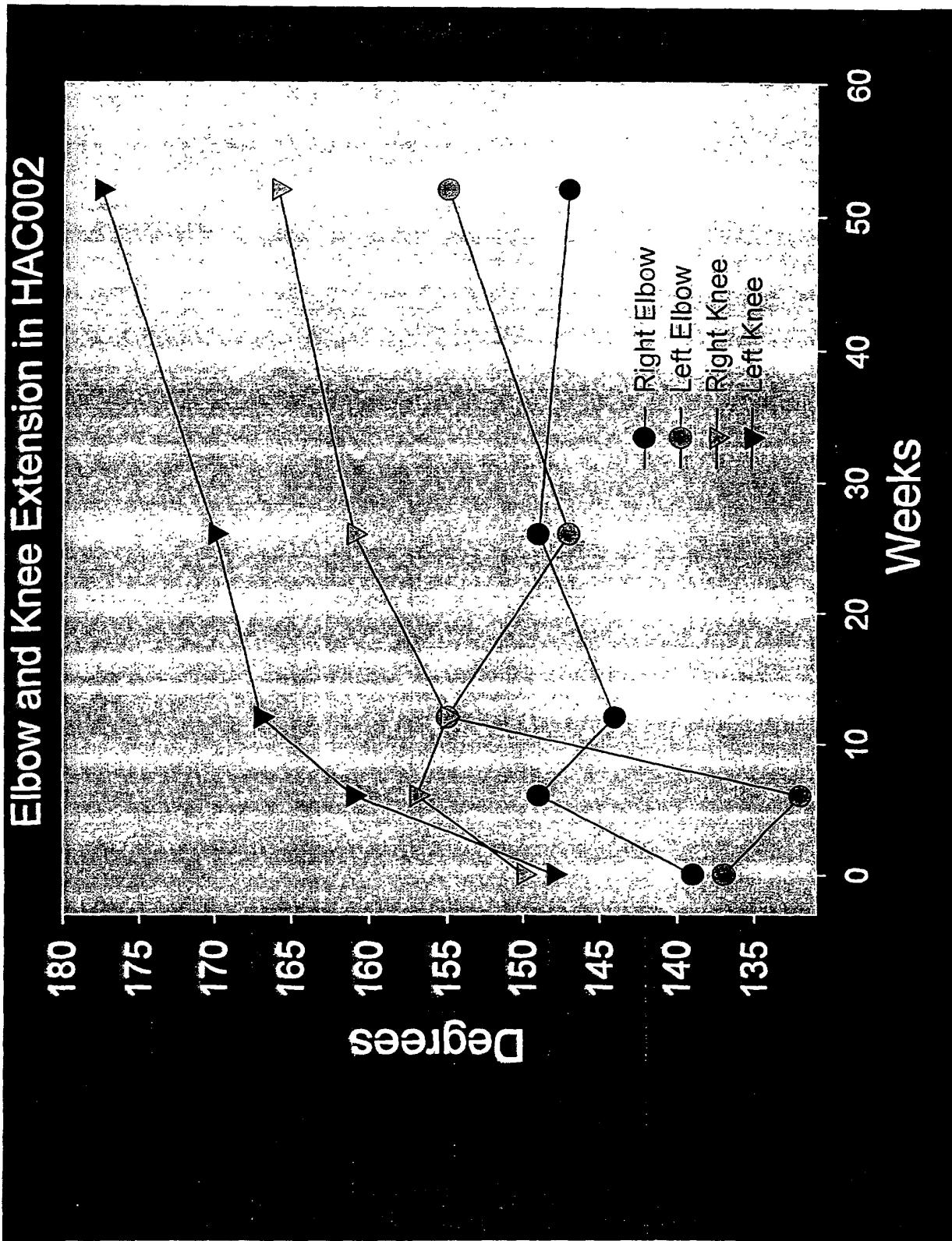


FIGURE 6

Shoulder flexion to 104 weeks in four patients with most restriction

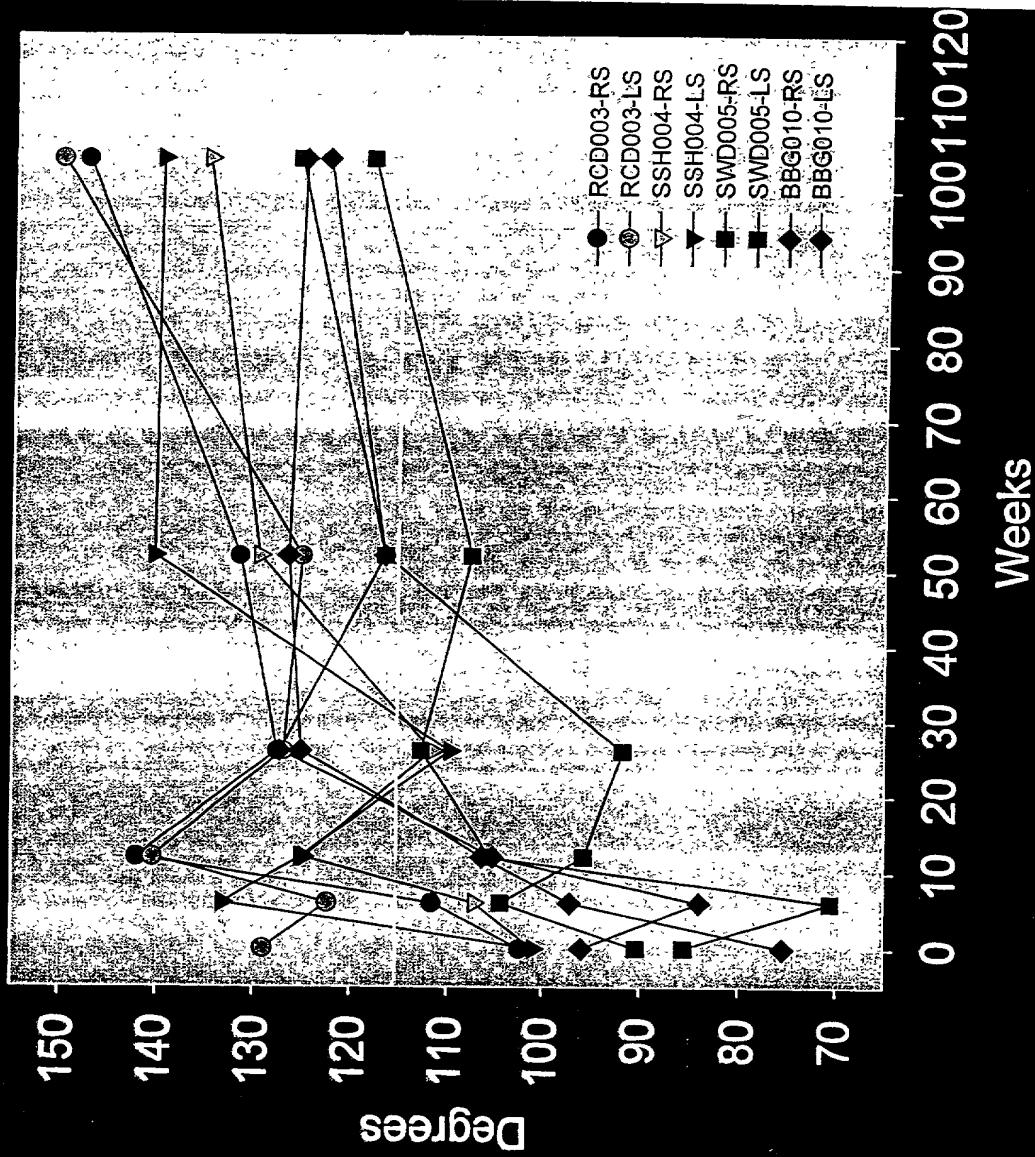


FIGURE 7

Sleep Apnea Improves

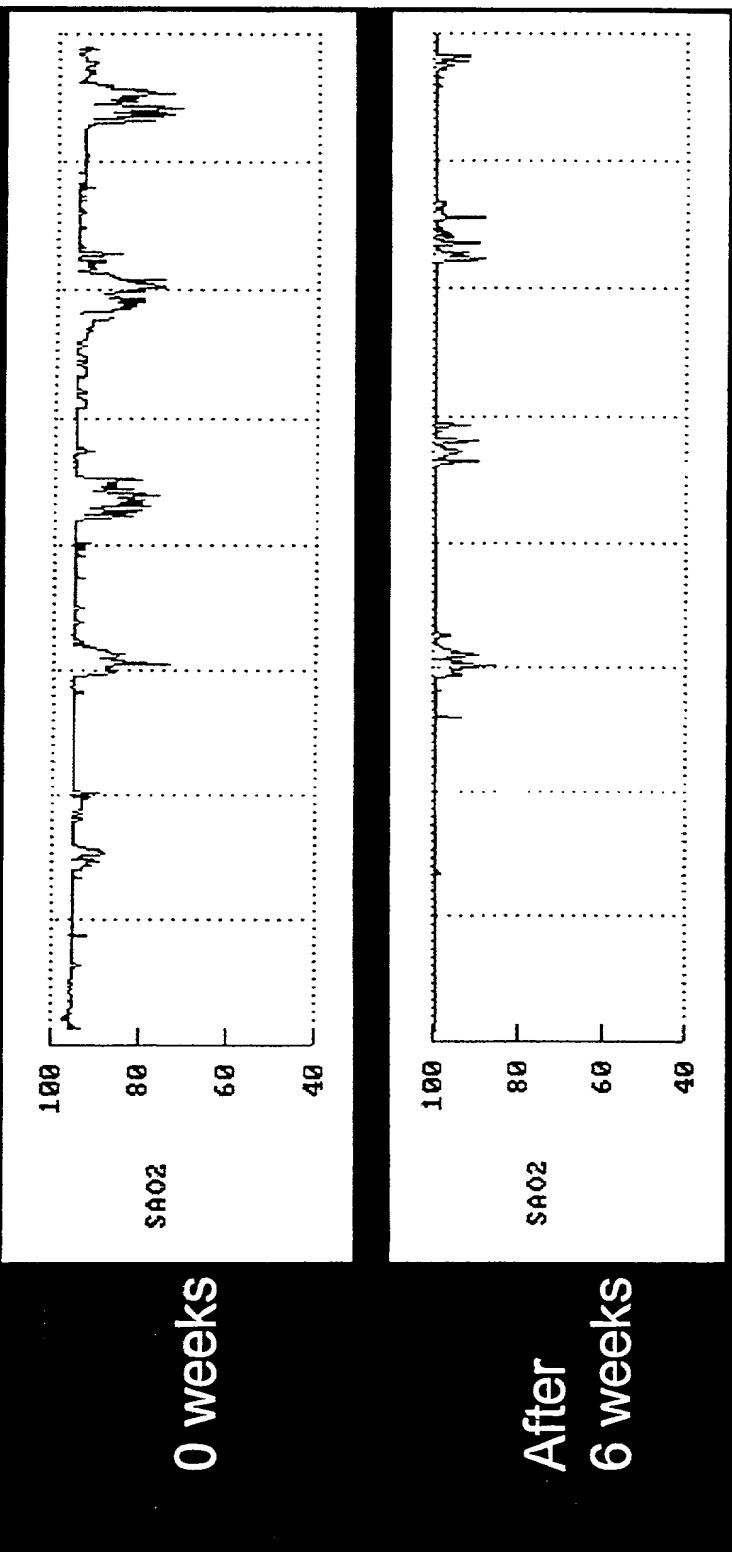


FIGURE 8

2003-01-17 06:00

Apneas + Hypopneas During Sleep Pre and Post Treatment

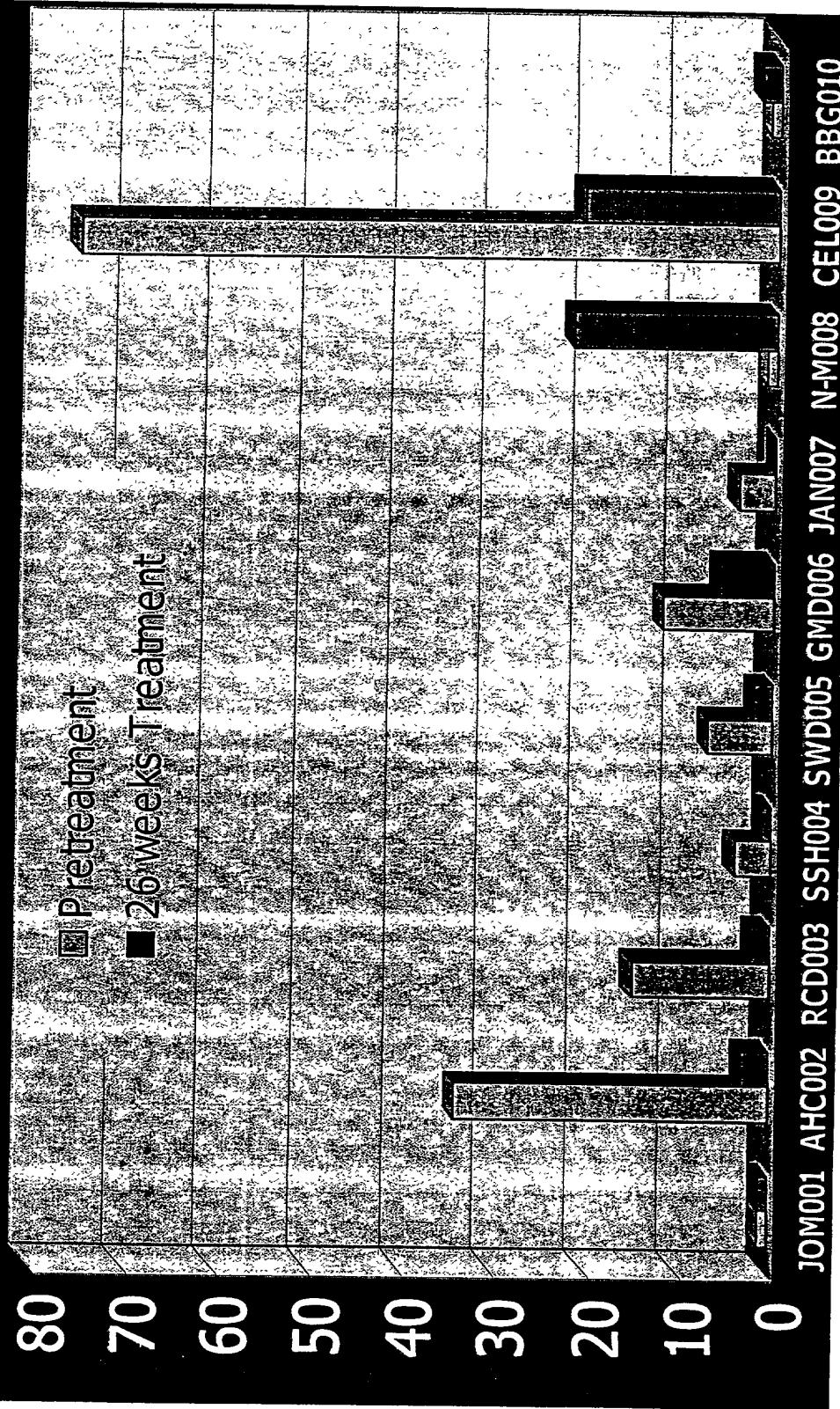


FIGURE 9

Pulmonary Function Tests in GMD006

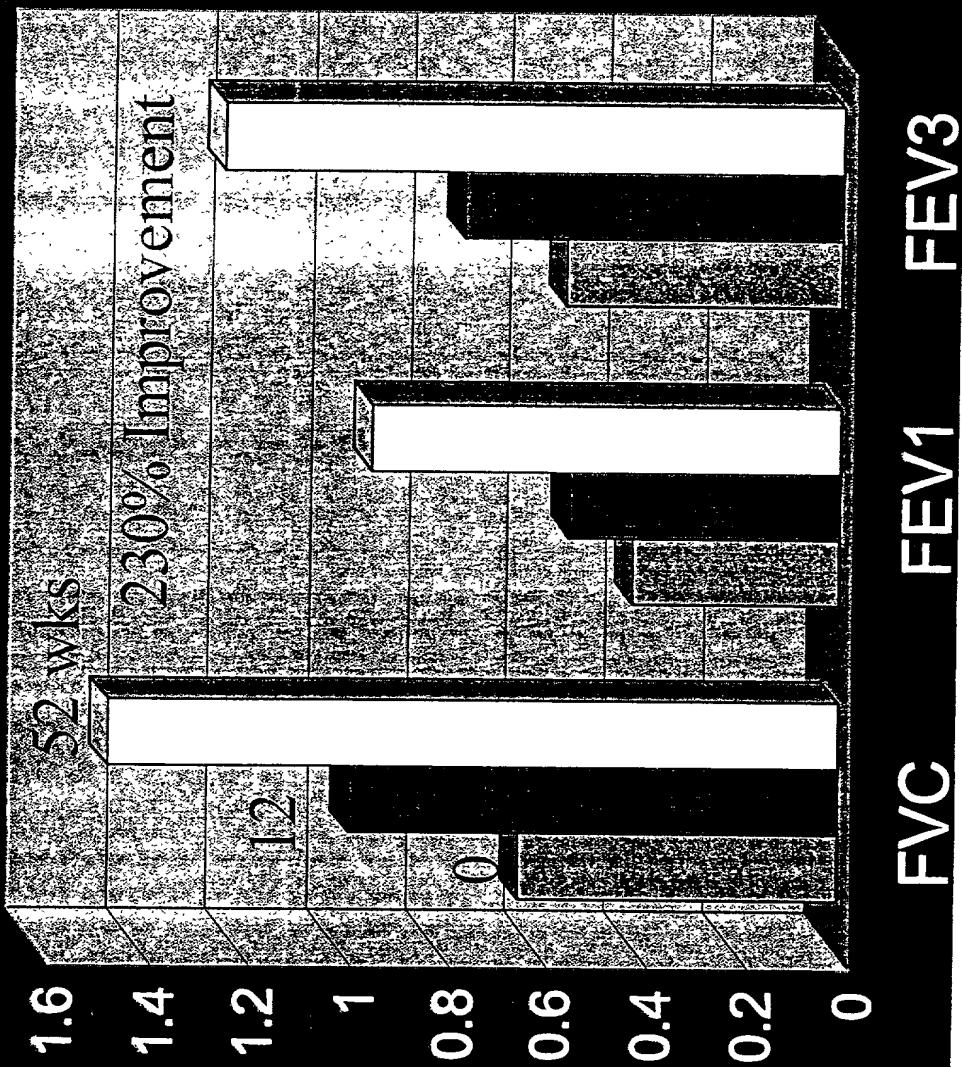


FIGURE 10

Increased Height Growth Velocity

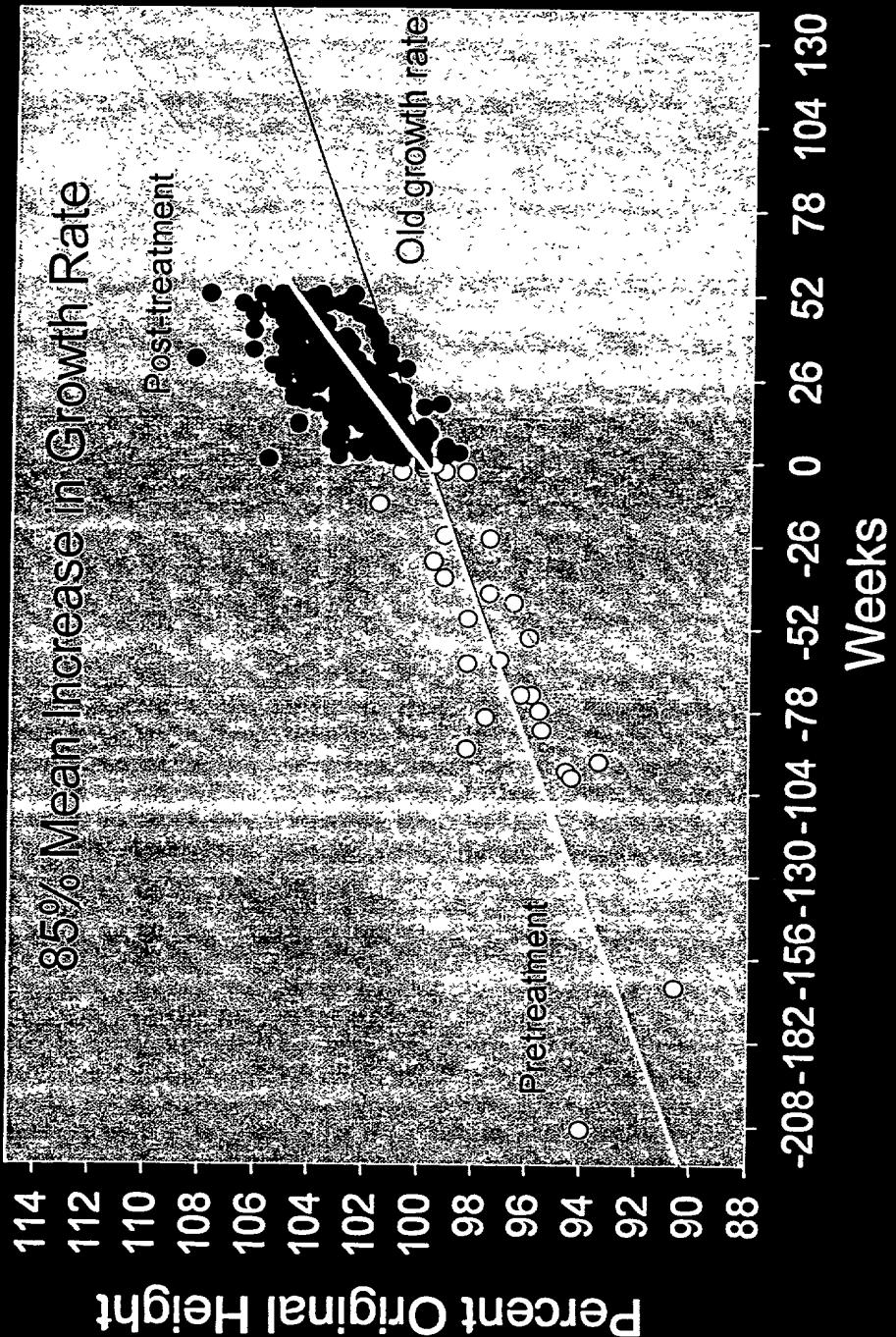


FIGURE 11

FIGURE 12.

COMPARISON OF HOST PROTEIN CONTAMINATION BETWEEN A PRIOR AND THE NEW GALLI PROCESS

Chinese Hamster Ovary Host Protein Contamination by ELISA Assay

SOURCE AND BATCH NUMBER	CHOP PROTEIN CONTAMINATION (microgram per milligram)	PERCENT CHOP CONTAMINATION	PURITY OF THE ENZYME FROM CHOP
Prior Process (Carson/REI)			
C9002	14	1.4%	98.6%
C9003	24	2.4%	97.6%
C9004	16	1.6%	98.4%
New Process (Galli)			
P1003	<1.3	<0.13%	>99.9%
P1006	1.2	0.12%	99.9%
P1007	<0.6	<0.06%	>99.9%
P1008	<0.67	<0.067%	>99.9%

FIGURE 12

Comparison of Galli and Carson Material

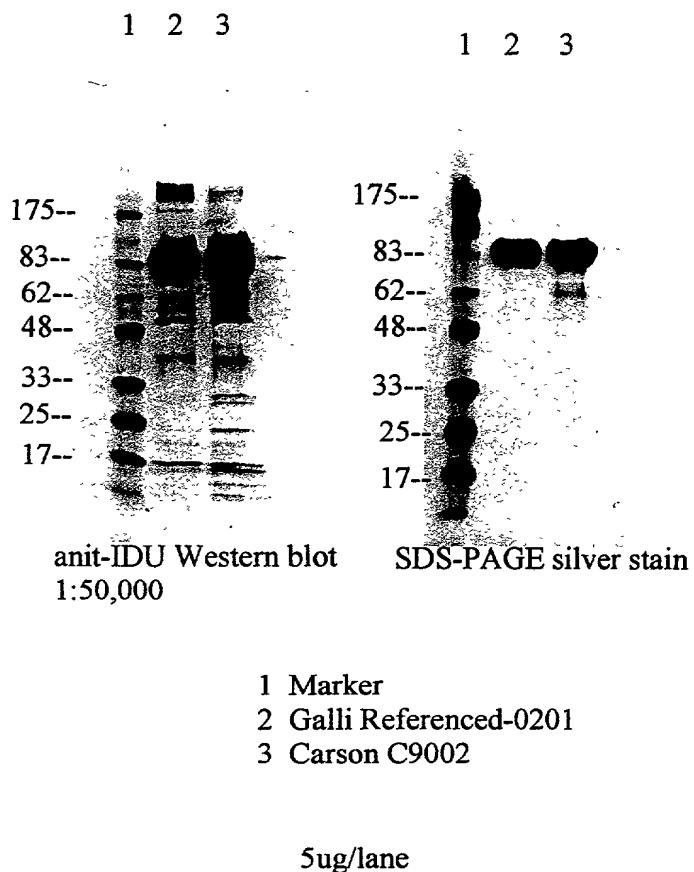


FIGURE 13